PCN172

A CALL TO MONITOR DRUG SHORTAGES AND THE ROLE OF MARKET ATTRACTIVENESS IN EUROPEAN COUNTRIES

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OBJECTIVES: Drug shortages are a global problem. While extensively studied in the United States, numbers about drug shortages in European countries are scarce. This study aims to investigate publically available data about drug shortages in European countries in order to reveal a typology of drug shortages in Europe. **METHODS:** A standardized reporting template was designed based on a literature search to collect and structure information. Countries offering an online reporting system for drug shortages such as Belgium, The Netherlands, England, Italy, France, Germany and Spain are included in this study. The online reporting systems were consulted in May 2013. Typology and causes of drug shortages are mapped and a sub-analysis is performed for essential medicines and oncology drugs. **RESULTS:** Majority of drugs reported to suffer from shortage (n=671) are branded (61%), oral drugs (51%) that equally affect different disease domains. When considering essential medicines (n=200) and oncology drugs (n=71), generics (55% for essential drugs, 64% for oncology drugs) and injectables (52% for essential drugs, 79% for oncology drugs) are more involved. Causes for drug shortages are underreported, as the cause is not known in 66% of the cases (n=671). Production problems are reported in 27% of the cases (n=671). Results are subjected to the different scopes of the considered reporting systems. **CONCLUSIONS:** Reporting of drug shortages in Europe needs to be standardized and more transparency about the reasons for drug shortages is required to understand the problem. A link between production problems and market attractiveness and market capacity is recognized to be at the root of drug shortages in U.S. Such insights are highly lacking in Europe. Monitoring of the effect of national and European health policies on the sustainability of the drug market is required to present fundamental solutions for the problem of drug shortages in Europe.

PCN173

DOWNFALLS OF THE FDA ACCELERATED APPROVAL PATHWAY – STRINGENT CONTROLS MUST BE INVOKED TO ENSURE PROMPT SUBMISSION OF FOLLOW-UP CONFIRMATORY TRIAL DATA

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OBJECTIVES: Ponatinib was temporarily withdrawn by the FDA in October 2013 following safety concerns arising from its Phase III trial. This drug had previously been approved under the accelerated approval pathway. Three other oncologics have been withdrawn under similar conditions, further adding to concerns with this pathway. This research aims to provide an up-to-date systematic analysis of all oncologics approved under this pathway and analyse the time delay in obtaining regular approval. **METHODS:** Publically available assessments of any oncologic approved under the FDA accelerated approval pathway were sourced and the dates of accelerated approval and conversion to regular approval were extracted. **RESULTS:** 41 oncologics across 50 indications have been assessed under the FDA accelerated approval pathway, all but two of which have been approved. Of the approved indications, 50% (24/48) have been converted to regular approval with an average delay of 53 months (range 13-151 months). 6% (3/48) have been withdrawn from the market due to lack of efficacy and/or safety concerns arising from Phase III data. 44% (21/48) have not been converted to regular approval despite being on the market for an average of 45 months (range 4–109 months). In these cases the mandatory confirmatory trials have not been completed and to date the FDA has not withdrawn a single oncologic from the market for not conducting the confirmatory trial. **CONCLUSIONS:** 21 oncologic indications approved under the accelerated approval process have not been converted to regular approval despite some being on the market for up to 9 years. Given that 11% (3/27) of the drugs that have conducted confirmatory trials have been withdrawn, completion of confirmatory data should become a strict, non-negotiable requirement with a defined time limit by which the data must be submitted. A failure to do so should see the FDA automatically withdrawing their license.

PCN174

EVIDENCE FOR A LOWERED THRESHOLD FOR FDA APPROVAL OF ONCOLOGICS BASED ON SINGLE-ARM PHASE II DATA, COMPARED TO THE EMA

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OBJECTIVES: The European Medicines Agency (EMA) approved 19 oncologics across 25 indications on the basis of pivotal Phase II data lacking an active comparator (Macaulay, ISPOR Dublin 2013). Approval was typically granted for indications in which there was no therapeutic alternative where a response rate of ≥35% was demonstrated. This research aims to define the circumstances under which the Food and Drug Administration (FDA) will approve oncologics on the basis of pivotal Phase II data and compare to those of the EMA. **METHODS:** A systematic search was undertaken for FDA oncologic submissions based on pivotal Phase II data and the acceptance decision, indication, and level of benefit were extracted. **RESULTS:** 31 oncologics across 38 indications were submitted to the FDA on the basis of pivotal Phase II data. All of which were non-comparative and 36 were approved. This included all drugs approved by the EMA on this basis except trabectidin. 32 indications were approved under the accelerated approval pathway, only 47% (15/32) of which have been converted to regular approvals. Two of these drugs have been subsequently withdrawn from the market as they failed to show benefit in confirmatory trials (gefitinib and gemtuzumab), neither of which were EMA-approved for these indications. 72% (23/32) were FDA designated orphan indications. 78% (25/32) indications were for lines of therapy or diseases that had no relevant therapeutic alternatives. The response rates of approved drugs ranged from 11%-86%, while 13 indications were approved with response rates of <35%, which included the 2 withdrawn drugs. **CONCLUSIONS:** Pivotal Phase II data can support FDA oncologic approvals for indications that lack therapeutic alternatives and demonstrate response rates of ≥10% (versus ≥35% for the EMA). The lower threshold enables more drugs for severe diseases to become available earlier in their development cycle but risks approving ineffective and/or unsafe drugs.

PCN175

A RETROSPECTIVE STUDY OF PATIENTS OUT-OF-POCKET COSTS FOR ORAL ONCOLOGY MEDICATIONS FOR MULTIPLE MYELOMA

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OBJECTIVES: To study patient's out-of-pocket expenditures in patients taking oral oncology medication for the treatment of Multiple Myeloma who are enrolled in a specialty pharmacy program. **METHODS:** A retrospective analysis of pharmacy claims and reimbursement data for oncology patients enrolled in a specialty pharmacy program and receiving biologic drugs from January 1, 2013 through October 31, 2013 was conducted. Patients with a primary diagnosis of Multiple Myeloma (ICD-9 CM: 203.xx) prescription data were included. There were no exclusion criteria. The distribution of out-of-pocket patient's costs per prescription were performed comparing average co-pay responsibility per prescription after insurance to average patient co-pay per prescription after funding assistance. **RESULTS:** A total of 22,566 prescriptions were included. The average patient co-pay responsibility after insurance was $435.00 per prescription and the average patient co-pay after funding assistance was $81.00 per prescription. This resulted in 12,822 (91.17%) of the prescriptions had a patient co-pay of under $10.00 after funding assistance. The patient's insurance type was as follows: private insurance was 59%, Medicare was 25%, Pharmacy Benefit Manager was 10%, Tricare was 1%, and Medicaid was 5%. **CONCLUSIONS:** In this retrospective analysis of pharmacy and financial claims data, Multiple Myeloma patients significantly reduced their out-of-pocket expenditures, from an average of $435.00 to $81.00 by the specialty pharmacy gaining funding assistance for the patient.

PCN176

ASSESSMENT OF IMAGING UTILIZATION AND TREATMENT PATTERNS FOR HEAD AND NECK CANCER PATIENTS IN THE UNITED STATES

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OBJECTIVES: To assess imaging and treatment patterns in head and neck cancer (HNC) patients using a large commercial-insurance database from the United States (U.S.). **METHODS:** We used the Marketscan® Research Databases (2007-2011) to identify adults with HNC (oral, pharynx, paranasal sinus, larynx) using ICD-9 codes. We evaluated three periods of imaging and treatment patterns: 1) three months pre-diagnosis, 2) diagnosis-to-treatment initiation, and 3) post-treatment initiation. Patients receiving single-imaging modalities and multiple-imaging modalities were evaluated in relation to surgery, chemotherapy, radiation therapy, and combinations. Imaging and treatment intensity and variability by cancer types and geographic regions (Northeast, North Central, South, and West) were assessed using multinomial and multivariate logistic regression. **RESULTS:** 80,987 patients were analyzed (39% female, mean age: 60 years). During pre-treatment, comparing all cancer types to oral cancer, pharynx cancer patients had the greatest likelihood of single-modality imaging and multiple-modality imaging. Patients with higher comorbidity index scores were more likely to receive more intensive imaging prior to treatment. Pre-treatment imaging was more likely to occur in other regions compared to West (OR range: 1.07-1.29), with consistent imaging patterns versus the West following treatment. There was limited regional variability in single and/or multiple intervention patterns. In the post-treatment period, patients receiving multiple treatment interventions, a proxy for advanced cancer, were more likely to undergo PET/CT. A high portion of larynx cancer patients received surgery (37%). Pharynx cancer patients were more likely to receive radiation therapy (24%) and/or chemotherapy (30%). During all phases combined, females were less likely to get imaging of any type (x-ray, CT, or PET/CT) (OR range: 0.71-0.91). **CONCLUSIONS:** Commercially-insured HNC patients in the U.S. vary in imaging intensity and in the types of imaging modalities used, prior to and following initial diagnoses. Receiving multiple treatment interventions was associated with undergoing multiple imaging tests and more specifically, PET/CT.

PCN177

DESCRIPTIVE ANALYSIS OF PATIENTS INITIATING REGORAFENIB THERAPY

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OBJECTIVES: To describes treatment patterns among patients initiating regorafenib, an oral kinase inhibitor indicated for the treatment of metastatic colorectal cancer in patients who have tried other first-line therapies. **METHODS:** Pharmacy and medical claims from Humana, a large national U.S. payer, were used. The study sample included patients age 19 to 89 years with at least one claim for regorafenib between 9-27-2012 and 6-1-2013. A subset of patients with pharmacy and medical benefits, as well as pre-index continuous enrollment of at least 12 months, was used to examine prior exposure to chemotherapy, radiation, and biologic therapies. Patients were followed until death, disenrollment or study end date (10-31-2013). **RESULTS:** A total of 407 patients with claims for regorafenib were identified. The mean age was 66.4 years, 53.1% were male, and median length of follow up was 140 days (range of 0-357 days). Median length of pre-index continuous enrollment was 779 days. The majority resided in the southern (51.6%) and midwestern (26.0%) U.S. and most patients had Medicare Advantage (26.0%) or Medicare Part D (69.3%) coverage. A total of 91 regorafenib patients met all inclusion/exclusion criteria. Metastatic cancer diagnosis was observed in 93.4% of patients; the majority had liver metastases. Common pre-index comorbidities included hypertension (72.5%), fluid/electrolyte disorders (41.8%), chronic pulmonary diseases (25.3%), diabetes (34.1%), and depression (15.4%). Evidence of chemotherapy, biologic therapy, and